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## **Stem Cell Agency Invests in Clinical Trial that Uses a One-Two Punch to Confuse and Kill Leukemia Cells**

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### **CIRM Board Also Supports Developing a Better, Safer Alternative to Bone Marrow Transplant**

**August 24, 2017 Oakland, CA** Our immune system is designed to protect us against infection and disease so when it goes wrong, as in cancer, we can be in serious trouble. Today the California Institute for Regenerative Medicine (CIRM), the state's stem cell agency, approved funding for a clinical trial that targets the most common form of leukemia in adults.

Chronic lymphocytic leukemia (CLL) is a type of blood cancer in which the bone marrow makes abnormal lymphocytes, a kind of white blood cell that is a key part of our immune system. This impairs our body's ability to fight infection and allows the cancer to spread, affecting the blood and bone marrow. Treatments may help slow down its progress but don't completely eradicate the cancer.

The CIRM Board is investing \$18.29 million in a clinical trial being run by Thomas Kipps, M.D., Ph.D., of the University of California, San Diego. He is using a combination strategy to kill the cancer stem cells that help CLL – and other cancers – survive traditional therapies, such as chemotherapy, and cause a relapse.

The therapy involves Ibrutinib, a drug already approved by the US Food and Drug Administration to treat CLL, which blocks signaling pathways that leukemia cells need to survive. Disrupting these pathways confuses the leukemia cell, leading to its death. But even with this approach there are cancer stem cells that are able to evade Ibrutinib. These cancer stem cells lie dormant during the therapy but come to life later, creating more leukemia cells and causing the cancer to spread and the patient to relapse. That's where the second therapy, cirmtuzumab, comes in. Cirmtuzumab (a monoclonal antibody developed with CIRM funding, hence the name) works by blocking a protein on the surface of the cancer stem cells that the cancer needs to spread.

It's hoped this one-two punch combination will kill all the cancer cells, increase the number of patients who go into complete remission and improve their long-term cancer control.

"Every year around 20,000 Americans are diagnosed with CLL" says Maria Millan, MD, interim President and CEO of CIRM. "For those who have run out of treatment options, the only alternative is a bone marrow transplant. Since CLL afflicts individuals in their 70's who often have additional medical problems, bone marrow transplantation carries a higher risk of life threatening complications. The combination approach of cirmtuzumab and Ibrutinib seeks to offer a less invasive and more effective alternative for these patients."

The CIRM Board also approved \$5 million for Angiocrine Bioscience Inc. to carry out a Phase 1 clinical trial testing a new way of using cord blood to help people who need a bone marrow transplant.

Many people battling deadly blood disorders need a bone marrow transplant but don't have a matched donor. Cord blood transplants provide an alternative source of transplant for these patients, helping rebuild and repair their blood and immune systems. However, too often a single cord blood donation does not have enough cells to treat an adult patient.

Angiocrine has developed a product that could help get around that problem. AB-110 is made up of cord blood-derived hematopoietic stem cells (these give rise to all the other types of blood cell) and genetically engineered endothelial cells – the kind of cell that lines the insides of blood vessels. This combination enables the researchers to take cord blood cells and greatly expand them in number. Expanding the number of cells could also expand the number of patients who could get these potentially life-saving cord blood transplants.

"Only around 30 percent of people who need a bone marrow transplant have a matched donor in their family," says Dr. Millan, "Many of the remaining 70 percent struggle to find an unrelated donor who is a match, particularly those with a racially or ethnically diverse background. Having an alternative way of offering people in need of a cord blood transplant could save many lives. This clinical trial will be the first step in determining if AB-110 is able to provide a way to improve the results of cord blood transplants."

## About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to [www.cirm.ca.gov](http://www.cirm.ca.gov)

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